

## CLAIMS

WHAT IS CLAIMED IS:

1. A cell carrying a recombinant adeno-associated viral (rAAV) vector, further  
5 comprising an exogenous gene encoding Apolipoprotein A-I (ApoA-I),  
ApoA-I<sub>Milano</sub>, a fragment of ApoA-I or ApoA-I<sub>Milano</sub>, or a derivative of ApoA-I or  
ApoA-I<sub>Milano</sub>.
2. The cell of claim 1, wherein said exogenous gene is ApoA-I<sub>Milano</sub>.
- 10 3. The cell of claim 1, wherein said rAAV vector is produced by the process of:  
(i) providing a first plasmid that comprises said exogenous gene,  
(ii) providing a second plasmid that is complementary to the first plasmid  
and which comprises components for rescue and packaging,  
15 (iii) co-transfecting the first and second plasmids into a host cell, and  
(iv) generating a quantity of said rAAV vector from said co-transfected  
host cell,  
wherein the pair of said first and second plasmids is selected such that  
said rAAV vector is targeted for delivery to a specific tissue type.
- 20 4. The cell of claim 3, wherein said second plasmid further comprises AAV rescue  
and packaging components derived from an AAV serotype selected from the  
group consisting of AAV1, AAV2, AAV5, AAV7, AAV8, AAV9, AAV10 and  
combinations thereof.
- 25 5. The cell of claim 1, wherein said cell is a stem cell.
6. The cell of claim 1, wherein said cell is a multipotent stem cell.
- 30 7. The cell of claim 1, wherein said cell is a bone marrow cell.
8. A recombinant adeno-associated viral (rAAV) vector, comprising an exogenous

gene encoding:

- (a) Apolipoprotein A-I (ApoA-I);
- (b) ApoA-I<sub>Milano</sub>;
- (c) A fragment of (a) or (b); or
- (d) A derivative of (a) or (b).

5

9. The vector of claim 8, wherein said exogenous gene is ApoA-I<sub>Milano</sub>.

10. The vector of claim 8, wherein said rAAV vector is produced by the process of:

10

- (i) providing a first plasmid that comprises said exogenous gene,
- (ii) providing a second plasmid that is complementary to the first plasmid and which comprises components for rescue and packaging,
- (iii) co-transfecting the first and second plasmids into a host cell, and
- (iv) generating a quantity of said rAAV vector from said co-transfected

15

host cell,

wherein the pair of said first and second plasmids is selected such that said rAAV vector is targeted for delivery to a specific tissue type.

11. The vector of claim 10, wherein said second plasmid further comprises AAV rescue and packaging components derived from an AAV serotype selected from the group consisting of AAV1, AAV2, AAV5, AAV7, AAV8, AAV9, AAV10 and combinations thereof.

20

12. A composition, comprising:

25

a recombinant adeno-associated viral (rAAV) vector, further including an exogenous gene encoding Apolipoprotein A-I (ApoA-I), ApoA-I<sub>Milano</sub>, a fragment of ApoA-I or ApoA-I<sub>Milano</sub>, or a derivative of ApoA-I or ApoA-I<sub>Milano</sub>; and a carrier.

30

13. The composition of claim 12, wherein said vector is formulated for delivery to a mammal.

14. The composition of claim 12, wherein said exogenous gene is ApoA-I<sub>Milano</sub>.

15. The composition of claim 12, wherein said rAAV vector is produced by the process of:
- (i) providing a first plasmid that comprises said exogenous gene,
  - (ii) providing a second plasmid that is complementary to the first plasmid and which comprises components for rescue and packaging,
  - (iii) co-transfecting the first and second plasmids into a host cell, and
  - (iv) generating a quantity of said rAAV vector from said co-transfected host cell,
- wherein the pair of said first and second plasmids is selected such that said rAAV vector is targeted for delivery to a specific tissue type.
16. The composition of claim 15, wherein said second plasmid further comprises AAV rescue and packaging components derived from an AAV serotype selected from the group consisting of AAV1, AAV2, AAV5, AAV7, AAV8, AAV9, AAV10 and combinations thereof.
17. A method of treating a condition in a mammal, comprising:
- providing a recombinant adeno-associated viral (rAAV) vector comprising an exogenous gene encoding Apolipoprotein A-I (ApoA-I), ApoA-I<sub>Milano</sub>, a fragment of ApoA-I or ApoA-I<sub>Milano</sub>, or a derivative of ApoA-I or ApoA-I<sub>Milano</sub>; and
  - delivering said rAAV vector to said mammal in an amount sufficient to treat said condition.
18. The method of claim 17, wherein said exogenous gene is ApoA-I<sub>Milano</sub>.
19. The method of claim 17, wherein said rAAV vector is produced by the process of:
- (i) providing a first plasmid that comprises said exogenous gene,
  - (ii) providing a second plasmid that is complementary to the first plasmid and which comprises components for rescue and packaging,
  - (iii) co-transfecting the first and second plasmids into a host cell, and
  - (iv) generating a quantity of said rAAV vector from said co-transfected host cell,
- wherein the pair of said first and second plasmids is selected such that

said rAAV vector is targeted for delivery to a specific tissue type.

20. The method of claim 19, wherein said second plasmid further comprises AAV rescue and packaging components derived from an AAV serotype selected from the group consisting of AAV1, AAV2, AAV5, AAV7, AAV8, AAV9, AAV10 and combinations thereof.
21. The method of claim 17, wherein said condition is selected from the group consisting of coronary heart disease, arterio-sclerosis, atherosclerosis, atherogenesis, vascular inflammation, release of cytokines or other secretory products at abnormal levels, suppression or aggravation of inflammatory or immunological response, and combinations thereof.
22. The method of claim 17, wherein said mammal is a human.
23. The method of claim 17, wherein said amount sufficient to treat said condition is from about  $1 \times 10^{10}$  vector genome/kg of said mammal to about  $1 \times 10^{14}$  vector genome/kg of said mammal.
24. The method of claim 17, wherein said vector is delivered to said mammal intramuscularly, intravenously, or both.
25. The method of claim 17, wherein providing said rAAV vector, further comprises transducing multipotent stem cells with a quantity of said rAAV vector, and delivering said rAAV vector further comprises transplanting said multipotent stem cells into said mammal.
26. The method of claim 25, wherein said cells are bone marrow cells.
27. A kit for the treatment of a disease condition in a mammal, comprising:  
a volume of recombinant adeno-associated viral (rAAV) vector comprising an exogenous gene encoding Apolipoprotein A-I (ApoA-I), ApoA-I<sub>Milano</sub>, a fragment of ApoA-I or ApoA-I<sub>Milano</sub>, or a derivative of ApoA-I or ApoA-I<sub>Milano</sub>; and instructions for the use of said volume of rAAV vector for treating a

condition in said mammal.

28. The kit of claim 27, wherein said exogenous gene is ApoA-I<sub>Milano</sub>.
- 5 29. The kit of claim 27, wherein said rAAV vector is produced by the process of:
- (i) providing a first plasmid that comprises said exogenous gene,
  - (ii) providing a second plasmid that is complementary to the first plasmid and which comprises components for rescue and packaging,
  - (iii) co-transfecting the first and second plasmids into a host cell, and
  - 10 (iv) generating a quantity of said rAAV vector from said co-transfected host cell,
- wherein the pair of said first and second plasmids is selected such that said rAAV vector is targeted for delivery to a specific tissue type.
- 15 30. The kit of claim 29, wherein said second plasmid further comprises AAV rescue and packaging components derived from an AAV serotype selected from the group consisting of AAV1, AAV2, AAV5, AAV7, AAV8, AAV9, AAV10 and combinations thereof.
- 20 31. The kit of claim 27, wherein said condition is selected from the group consisting of coronary heart disease, arterio-sclerosis, atherosclerosis, atherogenesis, vascular inflammation, release of cytokines or other secretory products at abnormal levels, suppression or aggravation of inflammatory or immunological response, and combinations thereof.
- 25 32. The kit of claim 27, wherein said mammal is a human.
33. The kit of claim 27, wherein said vector is transduced into multipotent stem cells.
- 30 34. The kit of claim 33, wherein said cells are bone marrow cells.
35. A method of preparing a rAAV vector, comprising:
- (i) providing a first plasmid that comprises an exogenous gene encoding Apolipoprotein A-I (ApoA-I), ApoA-I<sub>Milano</sub>, a fragment of ApoA-I or ApoA-I<sub>Milano</sub>, or

a derivative of ApoA-I or ApoA-I<sub>Milano</sub>;

(ii) providing a second plasmid that is complementary to the first plasmid and which comprises components for rescue and packaging,

(iii) co-transfecting the first and second plasmids into a host cell, and

5 (iv) generating a quantity of said rAAV vector from said co-transfected host cell,

wherein the pair of said first and second plasmids is selected such that said rAAV vector is targeted for delivery to a specific tissue type.